Public Workshop

Pediatric Clinical Investigator Training

Pooks Hill Marriot (Silver Spring, MD)
September 22, 2014

AGENDA (DRAFT)

"Pediatric Clinical Investigator Training"

8:00-8:10 am **Welcome and Introduction**

Dianne Murphy, M.D., F.A.A.P., Director, Office of Pediatric Therapeutics, FDA

- Goal and Overview of the Workshop
- FDA Requirements: They are different and why investigators need to know

---

Module I: Study Design and Beyond

8:10-9:10 am **1.1 FDA Perspective of Study Design and Plan**

Speaker: Lynne Yao, M.D. (CDER)

Discussion topics:

- Basic elements of successful trials: why open label trials are not FDA's standard and robust trials are needed.
- What is a well controlled trial?
- Issues of unvalidated endpoints in pediatrics
- Discussion : what is substantial evidence?
- Common errors made by clinical investigators from an FDA perspective
- Prelude to extrapolation

9:10-10:00 am **1.2 Extrapolation and Innovative Study Design**

Extrapolation (25 min)
Speakers: Hari Sachs, M.D. (CDER) and Dianne Murphy, M.D. (OC)

Innovative Study Design (25 min)
Speakers: Anne Pariser, M.D. (CDER) and Marc Walton, M.D. (CDER)

10:00-10:15 am **Break**

10:15-10:45 am **1.3 Ethics**

What do you need to know about special protections for pediatric subjects?

Speaker: Robert (Skip) Nelson, M.D. (OC)

10:45-11:25 pm **1.4 Get the Dosing Right**

What do we look for to support pediatric dosing? (20 min)
Speaker: Lily Mulugeta, PharmD. (CDER)

Experience from the PTN (20 min)
Speaker: Brian Smith, M.D., M.P.H, M.H.S., (Duke)

11:25-11:55 pm **1.5 Impacts of Pediatric Specific Safety Issues on Pediatric Trial Conduct**

Pre-marketing Section (15 min)
What Constitutes an Adequate Pediatric Safety Database?
Speaker: Linda Lewis, M.D. (CDER)

Post-marketing Section (15 min)
How Pediatric Specific Post-Market Safety Issues Impact on Pediatric Trial Conduct?
Speaker: Andy Mosholder, M.D. (CDER)

11:55-12:10 pm **1.6 Key Elements of Statistical Analyses for Studies with Small Populations (i.e. pediatrics)?**
Speaker: Lisa A. Kammerman, Ph.D. (AstraZeneca)

12:10-1:00 pm **Lunch** (on your own)

1:00-2:00 pm **Post – Module I Discussion**
Chairs: Dianne Murphy, M.D. and Lynne Yao, M.D.

Module II: Formulations, CMC and Inspections (GCP & GLP)

2:00-2:30 pm **2.1 Formulations and CMC**
Speaker: Julia Pinto, Ph.D. (CDER)

2:30-3:15 pm **2.2 How to Survive an FDA Inspection: What are GCP and GLP?**
Speaker: Janice Pohlman, (CDER)

3:15-3:30 pm **Break**

3:30-4:00 pm **Module III: Non-clinical Study(ies)**
Speaker: Melissa Tassinari, Ph.D. (CDER)

Module IV: Written Request Processes and NIH Real-life Experiences

4:00-4:15 pm **4.1 NIH Sponsored Written Requests for Products With and Without Remaining Exclusivity**
Speaker: Dianne Murphy, M.D. (OC)

4:15-5:00 pm **4.2 NIH Real Life Experiences**
Speaker: Anne Zajicek, M.D., PharmD. (NIH)

Discussion topics:
- High quality clinical trial designs directed at meeting FDA standards – Best Pharmaceuticals for Children Act experiences
- Need for reproducibility of scientific findings
- NIH/FDA product development activities directed to product labeling, publication of trial results and data availability
- Communication with regulators: what are the options, what types of meetings and interactions, expectations, goals and best practices
- Need for quality data that is packaged so it can be analyzed on a patient level and can be combined with other data.

5:00-5:15 pm **4.3 Orphan Drug Program and Network Collaboration**
Speaker: Jonathan Davis, M.D. (Tufts)

Discussion topics:
- FDA's Orphan Drug Program
- Networks and increasing integration and collaboration between academia, industry, FDA, NIH, and community groups

---

5:15-5:30 pm **Wrap-up**

Speakers: Dianne Murphy/Lynne Yao/Anne Zajicek/Jonathan Davis

---

5:30 pm **Adjourn**